

Wednesday, September 3

- 6:30 a.m. NIH security clearance at hotel lobby (Marriott Pooks-Hill)
- 7:00 a.m. Departure of bus from Marriott Pooks-Hill to the Cloisters at the NIH
- 7:30 a.m. Coffee and light refreshments
- 7:50 a.m. Opening remarks for conference and introduction to agenda on CRMO/SAPHO
Raphaela Goldbach-Mansky, M.D., Translational Autoinflammatory Disease Section, NIAMS, NIH, Bethesda, Maryland
Karyl Barron, M.D., Deputy Director, Division of Intramural Research, NIAID, NIH, Bethesda, Maryland
Stephen Groft, Pharm D., Director, Office of Rare Diseases at the NIH, Bethesda, Maryland
Dan Kastner, M.D., Ph.D., Clinical Director, NIAMS, NIH, Bethesda, Maryland
- 8:00 a.m. The clinical and genetic spectrum of CRMO/SAPHO and an introduction to mouse models
Polly Ferguson, M.D., Director, Pediatric Rheumatology University of Iowa, Iowa City
- 8:30 a.m. Pathogenesis and clinical presentation of SAPHO
Robert Inman, M.D., Director of the Arthritis Center of Excellence, University of Toronto, Toronto, Canada
- 8:50 a.m. Skin lesions in CRMO/SAPHO and clinical scoring of severity
Edward Cowen, M.D., Staff Clinician, Dermatology, NCI, NIH, Bethesda, Maryland
- 9:10 a.m. Majeed syndrome
Hatem El-Shanti, M.D., Director, Shafallah Medical Genetics Center, Doha, Qatar
- 9:30 a.m. DIRA, a new autoinflammatory disease with new insights into CRMO?
Raphaela Goldbach-Mansky, M.D., Translational Autoinflammatory Disease Section, NIAMS, NIH, Bethesda, Maryland
- 9:40 a.m. Coffee Break
- 10:00 a.m. Imaging of CRMO
Geetika Khanna, M.D., Professor of Radiology, University of Iowa, Iowa City, Iowa
- 10:20 a.m. Long term outcome of CRMO and clinical evaluation of the context of a clinical trial
Chantal Job-Deslandre, M.D., Professor of Rheumatology Hôpital Cochin, Paris, France

- 10:40 a.m. Evaluating and quantifying gastrointestinal disease
Colleen Hadigan, M.D., Head, Pediatric Gastrointestinal Consult Service, NIAID, NIH, Bethesda, Maryland
- 11:00 a.m. Treatment trials of CRMO
Ronald Laxer, M.D., CM, FRCPC, Professor of Divisions of Rheumatology, The Hospital for Sick Children, Toronto, Canada
- 11:20 a.m. Functional outcome measures in chronic diseases?
Scott Paul, M.D., Rehabilitation Department, Clinical Center, NIH, Bethesda, Maryland
- 11:40 a.m. Total body MRI in relapsing CRMO
Paivi Miettunen, M.D., Pediatric Rheumatology, Alberta Children's Hospital, Calgary, Canada
- 11.50 noon Lunch (Box lunch at the Cloisters)
- 12:30 a.m. Immunological evaluation of bone lesions
Deborah Novack, M.D., Assistant Professor, Pathology and Immunology, Washington University St. Louis, St. Louis, Missouri
- 12:50 p.m. Immunology of gastrointestinal and mucosal inflammatory lesions
Warren Strober, M.D., Chief, Mucosal Immunity Section, Laboratory of Host Defenses, NIAID, NIH, Bethesda, Maryland
- 1:10 p.m. Trapped receptors in TRAPS: a case study in understanding pathogenesis of an autoinflammatory syndrome
Richard Siegel, M.D., Ph.D., Head, Immunoregulation Unit, NIAMS, NIH, Bethesda, Maryland
- 1:30 p.m. Evaluating aberrant innate immune responses in clinical trials
Steven Holland, M.D., Chief, Laboratory of Clinical Infectious Diseases, NIAID, NIH, Bethesda, Maryland
- 1:50 p.m. Choosing Clinical trials Endpoints
Edward H. Giannini, MSc, DrPH, Professor, Division of Rheumatology at Cincinnati Children's Hospital, Cincinnati, Ohio
- 2:10 p.m. Coffee Break
- 2:30 p.m. **Concurrent Workshops**
- CLINICAL WORKSHOPS:
Bone imaging
Skin scoring
Entry criteria
Rheum and rehab endpoints

BIOMARKER WORKSHOPS:

Inflammation

Bone

GI

Please review the attached workshop assignment sheets for your workgroup assignment. If you do not find your name on this particular afternoon, please identify a discussion group you would like to participate in. Maps with assigned location for each workshop can be found at the reception area.

- 5:00 p.m. Meet with the entire group to discuss trial design and possible agents for intervention
- 6:30 p.m. Dinner at the Cloisters (only for those who registered upfront for this event)
- 8:00 p.m. Shuttle back to the hotel

Thursday, September 4

- 6:30 a.m. NIH security clearance at hotel lobby (Marriott Pooks-Hill)
- 7:00 a.m. Departure of bus from Marriott Pooks-Hill to the Cloisters at the NIH
- 7:30 a.m. Coffee and light refreshments
- 7:50 a.m. Welcome to newcomers for Behçet's meeting and discussion of scope of today's meeting
Raphaela Goldbach-Mansky, M.D., Translational Autoinflammatory Disease Section, NIAMS, NIH, Bethesda, Maryland
Karyl Barron, M.D., Deputy Director, Division of Intramural Research, NIAID, NIH, Bethesda, Maryland
Stephen Katz, M.D., Ph.D., Director, NIAMS, NIH, Bethesda, Maryland
- 8:00 a.m. Organizational and other help that CARRA can provide
Robert Fuhlbrigge, M.D., Ph.D., Boston Medical Center, Boston, Massachusetts
- 8:15 a.m. **Discussion of a clinical trial design and an international CRMO study consortium** (continuation from previous evening)
- 9:00 a.m. Setting up and maintaining a database for sharing clinical data
Lisa Rider, M.D., Environmental Autoimmunity Group, NIEHS, NIH, Bethesda, Maryland
- 9:15 a.m. Issues of setting up a pediatric Behçet's database, access from the US, compliance and possibilities to add functionality such as a clinical data repository.
Isabelle Koné-Paut, M.D., Department of Pediatrics and Pediatric Rheumatology, Hôpital de Bicêtre, Le Kremlin-Bicêtre, France
- 9:30 a.m. Specific issues of setting up a registry in patients with Behçet's disease: How it could work?
Yusuf Yazici, M.D., Assistant Professor Hospital for Joint Diseases, New York, NY
- 9:45 a.m. Oral lesions in inflammatory conditions, studying the immune response of oral lesions
Sharon Gordon, DDS, MPH, Ph.D., Department of Biomedical Sciences University of Maryland, Baltimore, Maryland
- 10:05 a.m. Coffee Break
- 10:15 a.m. Genetics of Behçet's disease and ongoing and planned research on the etiopathogenesis of the disease

Ahmet Gül, M.D., Professor of Rheumatology,
Istanbul University, Istanbul, Turkey

- 10:35 a.m. Ongoing research in children and adolescents with Behçet's disease
Emire Seyahi, M.D., Associate Professor of Rheumatology, Cerrahpasa Medical School, Istanbul, Turkey
- 10:55 a.m. Clinical spectrum of Behçet's disease in Japan and evaluation of novel biomarkers in the determination of disease subsets and response to therapy
Yoshiaki Ishigatsubo, M.D., Ph.D., Department of Medicine and Clinical Immunology, Yokohama City University, Yokohama, Japan
- 11:15 a.m. What we learnt from clinical trials in Behçet's disease
Hasan Yazici, M.D., Professor of Rheumatology,
Istanbul University, Istanbul, Turkey
- 11:35 a.m. JIA Heterogeneity as seen through gene expression analysis of peripheral blood components
Robert Colbert, M.D., Ph.D., Chief, Pediatric Translational Research Branch NIAMS, NIH, Bethesda, Maryland
- 11:55 a.m. Lunch (Box lunch at the Cloisters)
- 12:30 p.m. Abnormalities in the adaptive immune system
Graham Wallace, Ph.D., Senior Lecturer, Academic Unit of Ophthalmology University of Birmingham, Birmingham, United Kingdom
- 12.50 p.m. Detecting viruses in mucocutaneous lesions
Jeffrey Cohen, M.D., Chief, Medical Virology Section, NIAID, NIH, Bethesda, MD
- 1.05 p.m. Microarray analysis of blood samples
Ivona Aksentijevich, M.D., Staff Scientist, Genetics and Genomics Branch, NIAMS, NIH, Bethesda, Maryland
- 1.20 p.m. The genetic challenges in Behçet's disease
Elaine Remmers, Ph.D., Staff Scientist, Genetics and Genomics Branch, NIAMS, NIH, Bethesda, Maryland
- 1.35 p.m. Eye disease and outcomes in Behçet's disease
H. Nida Sen, M.D., MHS, Program Director, Uveitis and Ocular Immunology Fellowship, NEI, NIH, Bethesda, Maryland
- 1.50 p.m. An enlarging spectrum and a broader view of autoinflammatory diseases
Daniel Kastner, M.D., Ph.D., Clinical Director, NIAMS, NIH, Bethesda, Maryland
- 2:10 p.m. Coffee Break

2:30 p.m.

Concurrent Workshops

CLINICAL WORKSHOPS (Behcet's workshop 1):
Skin and mucosal scoring
Entry criteria
Rheum and rehab endpoints

BIOMARKER WORKSHOPS:
Vascular
Gastrointestinal
Inflammatory

Please review the attached workshop assignment sheets for your workgroup assignment. If you do not find your name on this particular afternoon, please identify a discussion group you would like to participate in. Maps with assigned location for each workshop can be found at the reception area.

6:00 p.m.

Leaving NIH for Dinner in Bethesda,
Shuttle will bring us from Cloisters to the Restaurant in Bethesda and
back to the Marriott-Pooks-Hill and NIH after dinner.

Dinner will be at the Cesco Trattoria in Bethesda

Cesco Trattoria
4871 Cordell Ave,
Bethesda, MD 20814
Tel. 301 654-8333

Friday, September 5

- 6:30 a.m. NIH security clearance at hotel lobby (Marriott Pooks-Hill)
- 7:00 a.m. Departure of bus from Marriott Pooks-Hill to the Cloisters at the NIH
- 7:30 a.m. Coffee and light refreshments
- 8:00 a.m. Opening remarks on Behçet's disease and potential trial design
Raphaela Goldbach-Mansky, M.D., Translational Autoinflammatory Disease Section, NIAMS, NIH, Bethesda, Maryland
- 8:10 a.m. Skin and mucosal pathology in Behçet's disease
Christos C. Zouboulis, M.D., Professor, Director Departments of Dermatology, Venereology, Allergology and Immunology, Dessau Medical Center, Dessau, Germany
- 8:30 a.m. Th17 and microbial stimulation, tests to evaluate adaptive and innate immune responses
Daniel Douek, M.D., Chief, Human Immunology Laboratory, Vaccine Research Center, Bethesda, Maryland
- 8:50 a.m. Determining ophthalmologic outcomes in clinical trials in Behçet's disease
Bahram Bodaghi, M.D., Ph.D., Professor of Ophthalmology, University of Paris, Paris, France
- 9:10 a.m. Evaluation of immunological processes in the skin and developing biomarkers in the context of an interventional study
Michelle A. Lowes, M.D., Assistant Professor of Clinical Investigation, The Rockefeller University, New York, New York
- 9:30 a.m. Coffee Break
- 9:45 a.m. **Concurrent Workshops**

CLINICAL WORKSHOPS (Behçet's workshop 2):

Skin and Mucosal Immunology

Ophthalmological Evaluation

Assessing "unmet needs" in Behçet's disease- insights from RCT

NATIONAL AND INTERNATIONAL COLLABORATIONS

Sharing clinical and biomarker data, study procedures and conducting clinical trials

Please review the attached workshop assignment sheets for your workgroup assignment. If you do not find your name on the Friday morning assignment sheet, please identify a discussion group you would like to participate in and join them. Maps with assigned location for each workshop can be found at the reception area.

12.00 p.m. Lunch (Box lunch Cloisters) Presentation and group discussions

1:30 p.m. Concluding Remarks and Adjourn